



CNS THERAPEUTICS THAT TARGET NEURONAL CEROID-LIPOFUSCINOSES AND THIOESTERASE DEFICIENCY DISORDERS

SUMMARY

The Eunice Kennedy Shriver National Institute of Child Health and Human Development seeks licensing or co-development partners for therapeutic compounds to treat lysosomal storage disorders.

REFERENCE NUMBER

E-157-2011

PRODUCT TYPE

- Diagnostics
- Therapeutics

KEYWORDS

- Non-toxic derivative of hydroxylamine
- Neuronal Ceroid-Lipofuscinoses (NCL)
- lysosomal storage disorders (LSD)
- Palmitoyl-protein thioesterase
- lysosome

COLLABORATION OPPORTUNITY

This invention is available for licensing and co-development.

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DESCRIPTION OF TECHNOLOGY

Clinically known as Neuronal Ceroid-Lipofuscinoses (NCL), Batten disease, is a rare neuron killing disease and one of the lysosomal storage disorders (LSDs). It is associated with a mutation or lack of palmitoyl-protein thioesterase-1 (PPT1) gene. It manifests very early in a child's life causing absence of brain activity as early as 4 years of age.

[Dr. Mukherje of NICHD](#) has discovered and developed N-t-BuHA, a chemical derivative of hydroxylamine that mimics the action of PPT1 enzyme. Compared to hydroxylamine, N-t-BuHA has been shown to be non-toxic in mice expressing batten disease. In addition, NtBuHA exhibited potent antioxidant property and extended the life of the diseased mice. NtBuHA has shown promising



therapeutic potential to treat NCL-LSDs.

POTENTIAL COMMERCIAL APPLICATIONS

- Small molecule therapeutic for neuronal ceroid-lipofuscinosis
- Small molecule to treat or prevent thioesterase deficiency disorders.

COMPETITIVE ADVANTAGES

- First of its kind to treat INCL and other LSD
- Non-toxic derivative therapeutic against thioesterase deficiency disorders

INVENTOR(S)

Anil Baran Mukherjee, Chinmoy Sarkar, Zhongjian Zhang (all of NICHD)

DEVELOPMENT STAGE

- Pre-clinical (in vivo)

PUBLICATIONS

Sarkar, C., et al. [[PMID 24056696](#)]

PATENT STATUS

- **U.S. Filed:** US Patent Application No. 14/110,393 filed 07 Oct 2013
- **Foreign Filed:** EP Patent Application No. 12716889.6 filed 07 Oct 2013

THERAPEUTIC AREA

- Central Nervous System, Mental and Behavioral, Pain